

## California's Stem Cell Agency Invests in Research Developing New Treatments for Parkinson's and Brain Tumors

Posted: June 28, 2018

**Oakland, CA** – The governing Board of the California Institute for Regenerative Medicine (CIRM), the state's stem cell agency, today voted to invest almost \$9.5 million to develop novel treatments for two devastating conditions of the brain.

John Zaia, M.D., at City of Hope, was awarded \$3.68 million to perform late stage preclinical research that would allow them to seek permission from the US Food and Drug Administration (FDA) to run a clinical trial targeting glioblastoma.

There is no cure for glioblastoma and even with aggressive surgery, chemotherapy and radiation, the median survival time is approximately 15 months (meaning half the patients survive this long). Because chemotherapy can cause severe toxic side effects, the dose of therapy that patients get is often limited.

The City of Hope team plans to address this limitation by genetically engineering the patient's own blood stem cells to protect them from chemotherapy and by sensitizing the tumor cells in a way that makes them more vulnerable to the chemotherapy. The goal is to allow patients to tolerate a higher dose of chemotherapy, to improve the patient's chances of responding to the chemotherapy and therefore of impacting survival and quality of life.

"Glioblastoma is the most common, and the most aggressive, form of brain cancer that led to the death of U.S. Senator Ted Kennedy and former Vice President Joe Biden's son Beau Biden," says Maria T. Millan, M.D. President and CEO of CIRM. "CIRM has supported a variety of stem cell-based approaches to target this devastating and currently untreatable condition. The project approved by our Board today is unique in that it seeks to use gene modified stem cells to allow patients to tolerate the high doses of chemotherapy while also making these tumors more susceptible to the chemotherapy."

The CIRM Board also awarded \$5.8 million to Krystof Bankiewicz, M.D., Ph.D., at the University of California, San Francisco (UCSF). In collaboration with Clive Svendsen, Ph.D. at Cedars-Sinai, this team is testing the potential for neural progenitor cells, engineered to express the growth factor GDNF, to impact Parkinson's disease. With CIRM funding, the investigators will perform pre-clinical research that is aimed at enabling them to file an application with the FDA to test this approach in a clinical trial.

Dr. Millan notes "CIRM is currently funding a Phase 1 clinical trial at Cedars-Sinai with this neural progenitor cell product for the treatment of ALS, another devastating neurodegenerative disorder for which there is no cure."

David Higgins, PhD, the CIRM Board Patient Advocate for Parkinson's disease said: "One of the big frustrations for people with Parkinson's, and their families and loved ones, is that existing therapies only address the symptoms and do little to slow down or even reverse the progress of the disease. That's why it's important to support any project that has the potential to address Parkinson's at a much deeper, longer-lasting level."

### About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With \$3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to [www.cirm.ca.gov](http://www.cirm.ca.gov)

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